Editas Medicine Announces Publication in Nature Medicine of Data Supporting the Development of EDIT-101 to Treat Leber Congenital Amaurosis 10 (LCA10)

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CAMBRIDGE, Mass., Jan. 21, 2019 (GLOBE NEWSWIRE) -- Editas Medicine, Inc. (Nasdaq: EDIT), a leading genome editing company, today announced the journal *Nature Medicine* published the comprehensive, pre-clinical data demonstrating the pharmacology and specificity of EDIT-101, an experimental, CRISPR genome editing medicine being investigated for the treatment of Leber congenital amaurosis 10 (LCA10), a genetically-driven form of blindness. EDIT-101 is set to be the first *in vivo,* or editing inside the body, CRISPR-based medicine administered to people anywhere in the world.

"We are thrilled *Nature Medicine* published our paper sharing the comprehensive set of pre-clinical data for EDIT-101 and our approach to creating CRISPR-based genome editing medicines," said Charles Albright, Ph.D., Chief Scientific Officer, Editas Medicine. "The research presented in this important manuscript provided the foundation for our recently accepted Investigational New Drug (IND) application for EDIT-101 to treat LCA10, bringing us closer to the clinic and our goal of realizing the full potential of genome editing. We look forward to initiating the Phase 1/2 study to further evaluate the safety, tolerability, and efficacy of EDIT-101 for people living with LCA10, a disease currently with no treatment options."

Published results detail the development of EDIT-101, an experimental genome editing medicine designed to remove the abnormal splice donor created by the IVS26 mutation in the CEP290 gene found in LCA10 patients and restore normal CEP290 expression. The paper summarizes *in vitro* experiments in human cells and retinal explants demonstrating the molecular mechanism of action and nuclease specificity.

Subretinal delivery of EDIT-101 in humanized CEP290 mice showed rapid and sustained CEP290 gene editing. A comparable surrogate non-human primate (NHP) vector also achieved productive editing of the NHP CEP290 gene at levels that met the target therapeutic threshold and demonstrated the ability of CRISPR/Cas9 to edit somatic primate cells *in vivo*. The results presented support further development of EDIT-101 for the treatment of patients with LCA10, as well as the application of genome editing approaches to treat a wide variety of inherited retinal diseases.

In the Phase 1/2 clinical trial, Editas Medicine and Allergan Pharmaceuticals International Limited (Allergan) plan to initiate patient screening mid-year and begin patient dosing in the second half of 2019, enrolling 10-20 patients in the U.S. and Europe.

About EDIT-101
EDIT-101 is a CRISPR-based experimental medicine under investigation for the treatment of Leber congenital amaurosis 10 (LCA10). EDIT-101 is administered via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells.

About Leber Congenital Amaurosis
Leber congenital amaurosis, or LCA, is a group of inherited retinal degenerative disorders caused by mutations in at least 18 different genes. It is the most common cause of inherited childhood blindness, with an incidence of two to three per 100,000 live births worldwide. Symptoms of LCA appear within the first years of life, resulting in significant vision loss and potentially blindness. The most common form of the disease, LCA10, is a monogenic disorder caused by mutations in the CEP290 gene and is the cause of disease in approximately 20-30 percent of all LCA patients.

About The Editas Medicine-Allergan Alliance
In March 2017, Editas Medicine and Allergan Pharmaceuticals International Limited (Allergan) entered a strategic alliance and option agreement under which Allergan received exclusive access and the option to license up to five of Editas Medicine's genome editing programs for ocular diseases, including EDIT-101. Under the terms of the agreement, Allergan is responsible for development and commercialization of optioned products, subject to Editas Medicine's option to co-develop and share equally in the profits and losses of two optioned products in the United States. In August 2018, Allergan exercised its option to develop and commercialize EDIT-101 globally for the treatment of LCA10. Additionally, Editas Medicine exercised its option to co-develop and share equally in the profits and losses from EDIT-101 in the United States. Editas Medicine is also eligible to receive development and commercial milestones, as well as royalty payments on a per-program basis. The agreement covers a range of first-in-class ocular programs targeting serious, vision-threatening diseases based on Editas Medicine's unparalleled CRISPR genome editing platform, including CRISPR/Cas9 and CRISPR/Cpf1 (also known as Cas12a).

About Editas Medicine
As a leading genome editing company, Editas Medicine is focused on translating the power and potential of the CRISPR/Cas9 and CRISPR/Cpf1 (also known as Cas12a) genome editing systems into a robust pipeline of treatments for people living with serious diseases around the world. Editas Medicine aims to discover, develop, manufacture, and commercialize transformative, durable, precision genomic medicines for a broad class of diseases. For the latest information and scientific presentations, please visit www.editasmedicine.com.

Forward-Looking Statements
This press release contains forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "target," "should,'' ''would,'' and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements in this press release include statements regarding the clinical trial timeline of EDIT-101. The Company may not actually achieve the plans, intentions, or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various factors, including: uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development of the Company's product candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial or the results of future trials; expectations for regulatory approvals to conduct trials or to market products and availability of funding sufficient for the Company's foreseeable and

unforeseeable operating expenses and capital expenditure requirements. These and other risks are described in greater detail under the caption "Risk Factors" included in the Company's most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission, and in other filings that the Company may make with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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